
Human Stem-Cell Based Development of a Potent Alzheimer's Drug Candidate

Grant Award Details

Human Stem-Cell Based Development of a Potent Alzheimer's Drug Candidate

Grant Type: Preclinical Development Awards

Grant Number: PC1-08086

Project Objective: To produce a pre-clinical package supporting further development for the neuroprotective small molecule CAD-31 in the Alzheimers indication.

Investigator:

Name: David Schubert

Institution: Salk Institute for Biological Studies

Type: PI

Disease Focus: Alzheimer's Disease, Neurological Disorders

Human Stem Cell Use: Embryonic Stem Cell, iPS Cell

Cell Line Generation: Embryonic Stem Cell, iPS Cell

Award Value: \$1,664,885

Status: Active

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Human Stem-Cell Based Development of a Potent Alzheimer's Drug Candidate

Public Abstract:

Over 6 million people in the US suffer from Alzheimer's disease (AD). There are no drugs that prevent the death of nerve cells in AD, nor has any drug been identified that can stimulate nerve cell replacement in aged human brain. Importantly, even if nerve cells could be replaced, the toxic environment of the AD brain which caused the disease in the first place will likely kill any cells that are born into that environment unless they are resistant to those conditions or can be protected by a drug. Therefore, drugs that stimulate the generation of new neurons (neurogenesis) alone will not be effective. A drug with both neurogenic and neuroprotective properties is required. With the ability to use cells derived from human neural precursor cells (hNPCs) derived from human embryonic stem cells (hESCs) as a screen for neurogenic compounds, we have shown that it is possible to identify and tailor drugs for therapeutic use in AD. With the support of CIRM, we have recently made a very potent AD drug candidate that is exceptionally effective in promoting the making of new nerve cells from human embryonic stem cells. It is both neurogenic and has therapeutic efficacy in a rodent model of AD. However, this molecule needs more preclinical development work before it can start the formal FDA pre clinical toxicity screening protocols. This work will optimize the chances for its true therapeutic potential in AD, and presents a unique opportunity to expand the use of hESCs for the development of a therapeutic for a disease for which there is no cure.

Statement of Benefit to California:

Over 6 million people in the US suffer from AD, and unless a viable therapeutic is identified it is estimated that this number will increase to at least 16 million by 2050, with a cost of well over \$1 trillion per year, likely overwhelming both the California and national health care systems. There is no treatment to prevent, cure or slow down this condition. In this application we have used the new human stem cell technologies to develop an AD drug candidate that stimulates the multiplication of nerve precursor cells derived from human embryonic stem cells. This approach presents a unique opportunity to expand the use of human embryonic stems cells for the development of a therapeutic for a disease for which there is no cure, and could lead to a paradigm shift in the treatment of neurodegenerative disease. Since our AD drug discovery approach is fundamentally different from the unsuccessful approaches used by the pharmaceutical industry. It could also stimulate new biotech. The work in this proposal addresses one of the most important medical problems of California as well as the rest of the world, and if successful would benefit all.

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